QOL in HTA decisions Dutch experience

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How can QOL contribute to decision making

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Conflict of interest

Nothing to declare

Content

- Soliris (eculizumab) in Paroxysmal Nocturnal Hemoglobinuria (PNH)
- Mammaprint in breast cancer (diganostic test)

Paroxysmal Nocturnal Hemoglobinuria

- A rare, acquired lifethreatening disease
- Destruction of red blood cells by the complement system (part of the immune system)
- Anemia and clot formation in blood vessels, leading to severe fatigue, iron deficiency
- Standard of care: frequent blood transfusion and anti-clotting agents
- Stem cell transplantation if a suitable donor is available
- Around 4000 patients in EU (0.1/10000)

Eculizumab

- The complement system (part of the immune system) has inflammatory properties and can destroy cells. Usually this system is activated as a defensive mechanism of the body.
- Eculizumab is an antibody that binds to one of the factors in the complement system called C5.
- Patients with PNH have an excess of C5 leading to destruction of red blood cells.
- Eculizamub binds to C5 and thus stops the breakdown of the red blood cells.

Eculizumab in Netherlands

- EU Registration as an orphan drug in 2007
- Conditional uptake in the reimbursement system in NL for 4 years
- 139 patients diagnosed in NL in the expert centre in Nijmegen
- Costs around € 360.000 per patient per year

- Re-evaluation in 2016-2017
- Product was considered marginally effective and but certainly not costeffective

Patient organisation involvement

- Company not willing to provide data from their registry
- Own survey by patient organisation
- 71 / 111 people completed the survey
- Employment rate in the eculizumab pats diagnosed after 2007 is comparable to the average in The Netherlands
- EQ5D-5L score is 0.796 (average in NL is 0.896)

Date of diagnosis	Nr of	On eculizumab	Working
Before 2007	24	16 (67%)	5 (31%)
After 2007	47	31 (67%)	18 (58%)

Result

ZINL (Dutch reimbursement agency) concluded

- Therapeutic added value demonstrated even for newly diagnosed patients (extension of orihginal indication for reimbursement)
- Still not cost effective (>80000 per qaly) so price negotiations needed
- For the time being product keeps its reimbursed status
- More information: info@aaenpnh.nl

Patient voice and QOL taken into account

Take home message

- Societal aspects of QOL have to be taken into account more
- Real patient involvement in HTA does help
- The non validated questionnaire on societal aspects was more helpful than the official validated E5QD-5L

Mammaprint (MP)

- Breast cancer patients with a high risk on recurrence are treated with adjuvant chemotherapy
- The risk is determined based on clinical symptoms
- Genetic profiling (MP) can be used to further specify the risk
- Around 25% of pats with clinical high risk are downgraded to low risk through MP

- MINDACT study is a prospective randomised trial to evaluate added value of mammaprint in preventing the use af adjuvant chemo

Results mindact
EunetHTA evaluation

- 5 yr progression free survival based on clinical evaluation is 94.5% as compared to 92% when based on mammaprint
- Side effects and QOL was not part of the MINDACT study so was difficult to evaluate

EunetHTA conclusion: no therapeutic added benefit

Patient organisation involvement (1)

NFK and Breastcancer organisation NL argued

- MP is not meant to increase progression free survival or overall survival, whatever you use the 5yr PFS is anyway high

HOWEVER

- Slightly lower 5yr PFS was reached with **23% less chemotherapy**
- Although adverse events and QOL is not measured the impact of chemotherapy is well known

Patient organisation involvement (2)

- MP is an instrument to determine an addtional cohort with low risk on recurrence

- In a process of shared decision making slightly lower 5yr PFS should be weighed against teh advantage of no chemotherapy
- Give the patients to change to make their own decisions

- Stop unnecessary treatment
- And possibly save money

Conclusion

- Remained negative
- Advice is to wait for 10yr overall survival data

Patient perspective and QOL data were NOT taken into consideration to an adequate level

Take home message

- Long time QOL (and side effects) are often not monitored
- They should however play an important role in evaluation

- OS is not always the most important to patients while I feel it still is the ONLY one for HTA

VRAGEN

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